

PTO/SB/08b (08-03)

Approved for use through 06/30/2006. OMB 0651-0031

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Substitute for form 1449A/PTO

**INFORMATION DISCLOSURE  
STATEMENT BY APPLICANT**

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Complete if Known

Application Number	10/552,287
Filing Date	April 18, 2004
First Named Inventor	Anthony FUTERMAN et al
Group Art Unit	1656
Examiner Name	Not Yet Assigned

Attorney Docket Number 30227

Sheet	1	Of	4
OTHER PRIOR ART – NON PATENT LITERATURE DOCUMENTS			
Examiner Initials	Cite No. <sup>1</sup>	Include name of the author (in CAPITAL LETTERS), title of the article (when appropriate), title of the item (book, magazine, journal, serial symposium, catalog, etc.) date, page(s), volume-issuc number(s), publisher, city and/or country where published.	T <sup>2</sup>
	1	Grabowski et al. "Human Acid $\beta$ -Glucosidase - Use of Conduritol B Epoxide Derivatives to Investigate the Catalytically Active Normal and Gaucher Disease Enzymes", The Journal of Biological Chemistry, 261(18): 8263-8269, 1986.	
	2	Berg-Fussman et al. "Human Acid $\beta$ -Glucosidase - N. Glycosylation Site Occupancy and the Effect of Glycosylation on Enzymatic Activity", The Journal of Biological Chemistry, 268(20):14861-14866, 1993.	
	3	Roeber et al. "Crystallization and Preliminary X-Ray Analysis of Recombinant Human Acid Beta-Glucocerbrosidase, A Treatment for Gaucher's Disease", Biological Crystallography, D59: 343-344, 2003.	
	4	Dvir et al. "X-Ray Structure of Human Acid- $\beta$ - Glucosidase, the Defective Enzyme in Gaucher Disease", The EMBO Journal, P.1-27, 2003.	
	5	Sawkar et al. "Chemical Chaperones Increase the Cellular Activity of N370S $\beta$ -Glucosidase:, A Therapeutic Strategy for Gaucher Disease", PNAS, 99(24): 15428-15433, 2002.	
	6	Erickson et al. "Biosynthesis of the Ltsomal Enzyme Glucocerebrosidase", The Journal of Biological Chemistry, 260(26): 14319-14324, 1985.	
	7	Ahn et al. "Crystal Structure of Saposin B Reveals A Dimeric Shell for Lipid Binding", Proc. Natl. Acad. Sci. USA, 100(1): 38-43, 2003.	
	8	Amaral et al. "Gaucher Disease: Expression and Characterization of Mild and Severe Acid $\beta$ -Glucosidase Mutations in Portuguese Type 1 Patients", European Journal of Human Genetics, 8: 95-102, 2000.	
	9	Amaral et al. "Type 1 Gaucher Disease: Identification of N396T and Prevalence of Glucocerebrosidase Mutations in the Portuguese", Human Mutation, 8: 280-281, 1996.	
	10	Beutler "Economic Malpractice in the Treatment of Gaucher's Disease", The American Journal of Medicine, 97: 1-2, 1994.	
	11	Beutler et al. "Gaucher Disease", The Metabolic and Molecular Bases of Inherited Disease, Chap.146: 3635-3668, 2001.	
	12	Beutler et al. "Two New Gaucher Disease Mutations", Human Genetics, 93: 209-210, 1994.	
	13	Brünger et al. "Crystallography & NMR System: A New Software Suite for Macromolecular Structure Determination", Acta Crystallographica Section D, 54: 905-921, 1998.	
	14	Buccoliero et al. "The Role of Sphingolipids in Neural Development: Lessons From Models of Sphingolipid Storage Diseases", Neurochemical Research, 27(7/8): 565-574, 2002.	

Signature	/David Steadman/	Considered	08/12/2008
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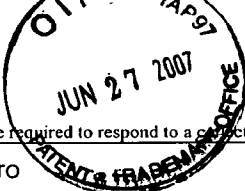
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	15	Charrow et al. "The Gaucher Registry. Demographics and Disease Characteristics of 1698 Patients With Gaucher Disease", Archive of Internal Medicine, 160: 2835-2843, 2000.			
	16	Chi et al. "Crystal Structure of the $\beta$ -Glycosidase From the Hyperthermophile Thermosphaera Aggregans: Insights Into Its Activity and Thermostability", FEBS Letters, 445: 375-383, 1999.			
	17	Cox et al. "Novel Oral Treatment of Gaucher's Disease With N-Butyldeoxynojirimycin (OGT 918) to Decrease Substrate Biosynthesis", The Lancet, 355: 1481-1485, 2000.			
	18	Davies et al. "Structures and Mechanisms of Glycosyl Hydrolases", Structure, 3: 853-859, 1995.			
	19	Dinur et al. "Human Acid $\beta$ -Glucosidase: Isolation and Amino Acid Sequence of A Peptide Containing the Catalytic Site", Proc. Natl. Acad. Sci. USA, 83: 1660-1664, 1986.			
	20	Fabrega et al. "Site Actif de la Glucocérébrosidase Humaine: Prédiction Structurale et Validations Expérimentales", Journal de la Société de Biologie, 196(2): 151-160, 2002. Article in French.			
	21	Fabrega et al. "Human Glucocerebrosidase: Heterologous Expression of Active Site Mutants in Murine Null Cells", Glycobiology, 10(11): 1217-1224, 2000.			
	22	Fan "A Contradictory Treatment for Lysosomal Storage Disorders: Inhibitors Enhance Mutant Enzyme Activity", Trends in Pharmacological Sciences, 24(7): 355-360, 2003.			
	23	De La Fortelle et al. "Maximum-Likelihood Heavy-Atom Parameter Refinement for Multiple Isomorphous Replacement and Multiwavelength Anomalous Diffraction Methods", Methods in Enzymology, 276: 472-494, 1997.			
	24	Futerman et al. The Cell Biology of Lysosomal Storage Disorders", Nature Reviews in Molecular & Cellular Biology, 5: 554-565, 2004.			
	25	Futerman et al. "New Directions in the Treatment of Gaucher Disease", Trends in Pharmacological Sciences, 25(3): 147-151, 2004.			
	26	Grabowski et al. "Enzyme Therapy for Lysosomal Storage Disease: Principles, Practice, and Prospects", Annual Reviews in Genomics & Human Genetics, 4: 403-436, 2003.			
	27	Grabowski et al. "Enzyme Therapy in Type 1 Gaucher Disease: Comparative Efficacy of Mannose-Terminated Glucocerebrosidase From Natural and Recombinant Sources", Annals of Internal Medicine, 122(1): 33-39, 1995.			
	28	Grabowski et al. "Acid $\beta$ -Glucosidase: Enzymology and Molecular Biology of Gaucher Disease", Critical Reviews in Biochemistry and Molecular Biology, 25(6): 385-414, 1990.			
	29	Grace et al. "Analysis of Human Acid $\beta$ -Glucosidase by Site-Directed Mutagenesis and Heterologous Expression", The Journal of Biological Chemistry, 269(3): 2283-2291, 1994.			

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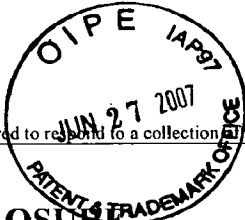
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31	Henrissat et al. "Updating the Sequence-Based Classification of Glycosyl Hydrolases", Biochemical Journal, 316: 695-696, 1996.		
32	Henrissat et al. "Conserved Catalytic Machinery and the Prediction of A Common Fold for Several Families of Glycosyl Hydrolases", Proc. Natl. Acad. Sci. USA, 92: 7090-7094, 1995.		
33	Hrmova et al. "Catalytic Mechanisms and Reaction Intermediates Along the Hydrolytic Pathway of A Plant $\beta$ -D-Glucan Glucohydrolase", Structure, 9: 1005-1016, 2001.		
34	Jones "A Graphics Model Building and Refinement System for Macromolecules", Journal of Applied Crystallography, 11: 268-272, 1978.		
35	Jones et al. "Improved Methods for Building Protein Models in Electron Density Maps and the Location of Errors in These Models", Acta Crystallographica Section A, 47: 110-119, 1991.		
36	Korkotian et al. "Elevation of Intracellular Glucosylceramide Levels Results in An Increase in Endoplasmic Reticulum Density and in Functional Calcium Stores in Cultured Neurons", The Journal of Biological Chemistry, 274(31): 21673-21678, 1999.		
37	Lachmann "Miglustat Oxford GlycoSciences/Actelion", Current Opinion in Investigational Drugs, 4(4): 472-479, 2003.		
38	Legler "Glucosidases", Methods in Enzymology, 46(Chap.40): 368-381, 1977.		
39	Legler "Glycoside Hydrolases: Mechanistic Information From Studies With Reversible and Irreversible Inhibitors", Advances in Carbohydrate Chemistry and Biochemistry, 48: 319-384, 1990.		
40	Lloyd-Evans et al. "Glucosylceramide and Glucosylsphingosine Modulate Calcium Mobilization From Brain Microsomes Via Different Mechanisms", The Journal of Biological Chemistry, 278(26): 23594-23599, 2003.		
41	Meivar-Levy et al. "Analysis of Glucocerebrosidase Activity Using N-(1-[14C]Hexanoyl)-D-Erythro-Glucosylsphingosine Demonstrates A Correlation Between Levels of Residual Enzyme Activity and the Type of Gaucher Disease", Biochemical Journal, 303: 377-382, 1994.		
42	Miao et al. "Identification of Glu340 as the Active-Site Nucleophile in Human Glucocerebrosidase by Use of Electrospray Tandem Mass Spectrometry", The Journal of Biological Chemistry, 269(15): 10975-10978, 1994.		
43	Mistry et al. "Therapeutic Delivery of Proteins to Macrophages: Implications for Treatment of Gaucher's Disease", The Lancet, 348: 1555-1559, 1996.		
44	Morel et al. "Effect of Mutations Within the Peripheral Anionic Site on the Stability of Acetylcholinesterase", Molecular Pharmacology, 55: 982-992, 1999.		

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